

CIRM Funded Clinical Trials

CNS10-NPC-GDNF delivered into the motor cortex for the treatment of ALS

Disease Area: Amyotrophic Lateral Sclerosis

Trial Sponsor: Cedars-Sinai Medical Center

Trial Stage: Phase 1

Trial Status: Launching

Targeted Enrollment: N/A

CIRM Awards Funding This Trial

Details:

ALS is a neurodegenerative disease that results in the death of nerve cells in the brain and spinal cord, causing the muscles in the body to gradually weaken, leading to loss of limb function, difficulty breathing, paralysis, and eventually death. There are medications that can slow down the progression of ALS, but unfortunately there is no cure for the disease. This trial uses a combined cell and gene therapy approach as a treatment for ALS and builds upon a CIRM funded ALS trial also conducted by Cedars-Sinai.

Genetically engineered stem cells will be transplanted into the motor cortex, an area of the brain responsible for voluntary movements. These transplanted cells then become astrocytes, a type of support cell that help keep nerve cells functioning. The astrocytes have been genetically altered to deliver high doses of a growth factor which has been shown to protect nerve cells.

Goal:

Protect the upper motor neurons controlling muscle function and meaningfully improve the quality of life for ALS patients.

Source URL: <https://www.cirm.ca.gov/clinical-trial/cns10-npc-gdnf-delivered-motor-cortex-treatment-als>